



**Roberta Brinton, Ph.D.**  
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Tucson, Arizona

2018 The Part the Cloud to RESCUE Brain Cell Degeneration in Alzheimer's Disease-  
\$1,000,000

## **Advancing Allopregnanolone as a Regenerative Therapeutic for Alzheimer's**

*This is a Phase 2a study to determine whether an important hormone in brain function is able to restore brain cell function in people with Alzheimer's.*

### **Background**

Allopregnanolone (or Allo) is a compound produced from the hormone progesterone. Allo is a type of steroid found in the brain that helps moderate the activity of nerve cells. Current research has found that Allo levels are reduced in people with Alzheimer's. Moreover, in studies with mice genetically engineered to develop Alzheimer's-like brain changes, Allo was shown to promote the generation of new nerve cells in the brain, to reduce levels of harmful beta-amyloid (a key molecular suspect in Alzheimer's), and to improve memory and other cognitive functions.

Dr. Roberta Brinton previously conducted a Phase 1 clinical trial with human participants demonstrating that Allo could prevent dementia-related shrinkage in the brain. These results identify Allo as a potential Alzheimer's drug — one that is both regenerative (able to replace lost brain cells) and protective against brain cell damage.

### **Research Plan**

Dr. Roberta Brinton and colleagues will conduct a Phase 2a clinical trial to test the safety and efficacy of the drug Allopregnanolone in 12 participants with Alzheimer's. Specifically, they will assess the effectiveness of injecting Allo into the muscle (intramuscular) versus direct injection into the veins. Dr. Brinton believes a drug formulation that enables intramuscular administration will ease clinical trial participation and in the long term facilitate broader clinical use of the drug, which can be more easily administered by a home nurse, pharmacist or a trained care-giver. The researchers will also evaluate how Allo therapy moderates a variety of factors linked to dementia, including loss of brain volume, decline in cognitive function and the ability to carry out daily tasks. Finally, Dr. Brinton will test the use of wearable sensors that can monitor daily activity and sleep patterns of the study participants over time.

### **Impact**

The results of this study could shed new light on optimal methods of administering the Allopregnanolone and how the drug affects the brain during Alzheimer's. If successful,

#### **PI**

- Ph.D. in Neuropharmacology and Psychobiology, University of Arizona, 1984
- Director, University of Arizona Center for Innovation in Brain Science
- Awarded the 2010 Presidential Citizens Medal for promoting scientific careers among minority students

#### **STUDY**

- CADRO category: Translational Research & Clinical Interventions
- Recipient of the Association's Sex and Gender in Alzheimer's (SAGA) grant that investigates why women are disproportionately affected by Alzheimer's.

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Dr. Brinton's effort may lead to a future Phase 3 clinical trial, in which Allo's potential benefits could be thoroughly tested in many more human volunteers.